



uniQure Announces Completion of Patient Enrollment in the First Two Cohorts of its Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

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LEXINGTON, Mass. and AMSTERDAM, March 21, 2022 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the completion of patient enrollment in the first two cohorts of its randomized, double-blinded, Phase I/II clinical trial of [AMT-130 for the treatment of early stage Huntington's disease](#).

"We are very pleased to have completed the enrollment of the first 26 patients in this ongoing clinical trial," stated Ricardo Dolmetsch, Ph.D., president of research and development. "It's a major milestone for the trial and an important achievement for our clinical operations team. Our investigators have done an exceptional job in enrolling this important study. We look forward to providing a clinical update in the second quarter of this year from the 12-month interim analysis of the 10 patients in the first cohort, including safety, mutant HTT protein (mHTT) and neurofilament light chain (NfL) data. We also remain on track with the enrollment of our European open-label clinical trial of AMT-130 and plan to begin a third patient cohort in the U.S. to explore an improved administration procedure."

The ongoing [Phase I/II clinical trial of AMT-130](#) is a randomized, sham controlled, double-blinded study to explore the safety, tolerability, and proof of concept of AMT-130 in patients with early manifest Huntington's disease. The study includes two dose cohorts of 26 patients randomized to either treatment with AMT-130 or to an imitation surgical procedure. The first dose cohort includes 10 patients, of which six patients received treatment with AMT-130 and four patients received imitation surgery. The second dose cohort includes 16 patients, of which ten patients received treatment with AMT-130 and six patients received imitation surgery. A third cohort exploring the use of an alternative stereotactic navigation system to simplify placement of infusion catheters will include up to 18 additional randomized patients. The Phase I/II clinical trial consists of a blinded 12-month period followed by unblinded long-term follow-up for five years. The treated patients have received a single administration of AMT-130 using [MRI-guided, convection-enhanced stereotactic neurosurgical delivery](#) directly into the striatum (caudate and putamen).

The ongoing Phase Ib/II trial of AMT-130 in Europe is an open-label study that will enroll 15 patients with early manifest Huntington's disease across two dose cohorts. Together with the U.S. study, the European study is intended to establish safety, proof of concept, and the optimal dose of AMT-130 to take forward into a pivotal study or into a confirmatory study should an accelerated registration be feasible.

AMT-130 comprises a recombinant AAV5 vector carrying a DNA cassette encoding a microRNA that lowers Huntingtin protein in Huntington's disease patients. AMT-130 is uniQure's first clinical program incorporating its proprietary miQURE[®] microRNA platform. miQURE is designed to degrade the messenger RNA produced by disease-causing genes without causing toxicity and to spread through an organ using exosomes.

About Huntington's Disease

Huntington's disease is a rare, inherited neurodegenerative disorder that leads to motor symptoms including chorea, and behavioral abnormalities and cognitive decline resulting in progressive physical and mental deterioration. The disease is an autosomal dominant condition with a disease-causing CAG repeat expansion in the first exon of the huntingtin gene that leads to the production and aggregation of abnormal protein in the brain. Despite the clear etiology of Huntington's disease, there are no currently approved therapies to delay the onset or to slow the disease's progression.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. We are leveraging our modular and validated technology platform to rapidly advance a [pipeline](#) of proprietary gene therapies to treat patients with hemophilia B, Huntington's disease, refractory temporal lobe epilepsy, Fabry disease, and other diseases. www.uniQure.com

uniQure Forward-Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, whether we will share mutant HTT protein (mHTT), neurofilament light chain (NfL), or other safety and biomarker data from any patients in our AMT-130 clinical trials during the second quarter of 2022, or ever, and whether we will initiate enrollment of a third cohort in our Phase I/II study of AMT-130 in 2022, or ever. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our clinical development activities, regulatory oversight, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in the Company's periodic securities filings, including its Annual Report on Form 10-K filed February 25, 2022. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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The logo for uniQure, featuring the word "uniQure" in a bold, orange, sans-serif font. The "Q" is stylized with a dot.